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Scalable production of gene therapy vectors: an interview with Frank Ubags

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Interview conducted by April Cashin-Garbutt, MA (Cantab)

insights from industry

Frank Ubags

CEO, CEVEC Pharmaceuticals GmbH

Can you please give a brief overview of gene therapy? How successful has it been so far?

Gene therapy involves introducing genetic material into cells to replace missing or malfunctioning genes or to add a gene to express a beneficial protein in order to treat certain diseases, such as cancer or inherited disorders.

Since the first gene therapy product was approved by regulatory authorities in the Western world in 2012, we are today in a good position for providing CAP®GT as the technology of choice for the commercials production of gene therapy vectors.

Almost 500 gene therapy candidates are currently in development. According to market reports, gene therapies targeting cancer alone could reach peak sales of $13 B by 2025.

No question that gene therapy is at the forefront of modern medical treatment. However, one of the biggest challenges in gene therapy today and still preventing this innovative approach from commercial use is the ability to reliably produce safe gene therapy vectors at an industrial scale. It is at this point that we are expecting to play a

key role with our innovative CAP®GT technology by providing a platform that exactly addresses the market needs allowing industrial scale production of gene therapy viral vectors.

Why is the production of gene therapy vectors on an industrial scale so challenging?

A major challenge is caused by the nature of the traditionally used expression systems.

The major current production platforms for viral gene therapy vectors are adherently growing cells like human embryonic kidney (HEK) 293 cell lines which are used in cell biology research and the biotechnology industry to produce gene therapy vectors. However, the scalability of adherent cell culture is limited.

Moreover, these cultures often require serum for efficient cell growth, which is undesirable from a regulatory standpoint. Attempts at suspension cultures of HEK 293 are being made, but they often prove to be a technological hurdle.

Growing as pure single cells in serum-free suspension cultures, our CAP®GT cells provide the highest cell densities, allow for easier scale-up and reduced production costs when compared to adherent cell culture systems.

What factors must be taken into account when producing a vector that is both reliable and safe?

From my point of view there are three main factors:

- A fully scalable system to produce flexible batch sizes from small scale to large industrial scale.
- Chemically defined, serum free culture conditions.
- A well-documented and safe expression host addressing all regulatory requirements.

Can you please outline CEVEC's new unique cell-expression system for the scalable production of gene therapy vectors?

The CAP®GT cell lines, derived from a non-tumor human origin, were developed with complete documentation and full certification of materials. As from the start, the development was aimed at the industrial scale production of gene therapy vectors.

Growing in serum-free suspension cultures, CAP®GT cell lines provide high cell densities of up to 2x10e7/ml in all common bioreactor formats significantly outperforming HEK 293 cells and show very high transfection efficiencies with common transfection reagents and methods.

CAP®GT cell lines do not produce undesired replication competent adenovirus (RCA). This was a main objective as from start of the development program. Especially for the field of adenoviral vector production we outperform HEK 293 in this respect.

What viral species do CAP®GT cells support?

CAP®GT cells show a broad viral propagation spectrum relevant for gene therapy applications including lentivirus (LV), adenovirus (AV) and adeno-associated virus (AAV).

In mid-September you announced a collaboration agreement in this with Généthon. Please can you outline CEVEC's partnership with this company?

Généthon is an important player with huge experience in developing gene therapies for rare diseases with several products in clinical development. The fact that they have chosen our CAP®GT system for the production of lentiviral vectors for gene therapy applications is a strong endorsement of our technology.

Généthon plans to develop lentivirus packaging cell lines to address the field of rare diseases with its own portfolio of gene therapy products. Furthermore, Généthon will manufacture CAP®GT derived lentiviral vectors for its partners and customers. CEVEC will grant the corresponding license to Généthon's partners for clinical trials and commercial production.

What further improvements can be made to CAP®GT technology?

We are currently working on so called packaging cell lines which would significantly facilitate the production process of gene therapy vectors, particularly in the lentiviral and AAV fields.

What do you think the future holds for the production of gene therapy vectors and how does CEVEC plan to contribute?

With the first products on the market and a huge pipeline of innovative products in clinical development, gene therapy is now well positioned to establish itself as a therapeutic option for a variety of diseases. The increasing number of available therapies and treatments will call for scalable industrial manufacturing of safe and efficient vectors including lentiviral, adenoviral and adeno-associated viral vectors on robust production platforms.

CEVEC's CAP®GT expression platform is a fully scalable production system for gene therapy vectors with broad regulatory endorsement. The superior features in terms of easy scale up of the CAP®GT suspension cell lines, production efficiency of the expression system and safety of CAP®GT-derived viral vectors position the CAP®GT technology at the cutting-edge of the rapidly growing field of viral vector industrial scale manufacturing for gene therapy applications.

Where can readers find more information?

For more information, please visit our website under www.cevec.com.

About Frank Ubags, CEO of CEVEC Pharmaceuticals GmbH

With over 35 years of leadership experience, Frank Ubags executed major financing and licensing transactions while instigating and driving company growth and turn around strategies. He initiated and executed several major M&A transactions in the Life Science sector, worldwide. Among others, he worked for Rhein Biotech, Kiadis Pharma and Scil Proteins in CEO, CFO and COO positions.